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Review Article

Neurodegenerative Disease Gene Therapy

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Abstract

Millions of patients with neurodegenerative disorders may receive therapeutic benefit from gene therapy through a variety of ways, including direct repair of pathogenic mechanisms, neuroprotection, neurorestoration, and symptom control. Therefore, understanding the illness pathophysiology and the necessary temporal and geographic specificity of gene expression are essential for effective therapeutic outcomes. The most thorough transduction of the target structure while preventing leaking into surrounding areas or perivascular spaces is another significant difficulty. In the newest technological era of gene therapy, interventional MRI-guided convection-enhanced delivery (iMRI-CED) is the gold standard for real-time verification of precise vector administration. The availability of this cutting-edge method of neurosurgery should hasten the development of potential preclinical treatments for neurodegenerative illnesses like Parkinson's, Huntington's, and Alzheimer's.

Keywords: Alzheimer's disease, Gene therapy, Huntington's disease, Parkinson's disease, Intraoperative MRI, Viral vector

INTRODUCTION

Gene therapy is a branch of medicine that focuses on altering a cell's genetic makeup to have a therapeutic impact or to treat a disease by replacing or repairing damaged genetic material (Flores Mireles AL., et al 2015). Although Martin Cline made the first effort to alter human DNA in 1980, the National Institutes of Health-approved first nuclear gene transfer in humans was carried out in May 1989. French Anderson carried out the first direct insertion of human DNA into the nuclear genome as well as the first therapeutic use of gene transfer in a trial that began in September 1990. Many genetic illnesses are expected to be curable or treatable with it over time (Andreu A., et al 2008). The original ideas for gene therapy emerged in the 1960s and early 1970s as genetically marked cell lines were being developed and the methods of cell transformation by the papaovaviruses polyoma and SV40 were being clarified (McIsaac WJ., et al 2011). Cloned genes were made available with the advent of recombinant DNA technology, and they were utilised to show that foreign genes may really treat genetic flaws and disease phenotypes in mammalian cells in vitro (Lewis JF., et al 1976). Effective retroviral vectors and other gene transfer techniques have enabled convincing in vitro and in vivo phenotypic correction demonstrations, making gene therapy a widely acknowledged therapeutic modality and supporting clinically relevant studies involving human subject (Manoni F., et al 2009). More than 20 years ago, the first gene therapy clinical studies were started. Gene therapy's early development was hampered by the occurrence of serious side effects in a small number of treated individuals, as was the case with many experimental medical techniques (Karakukcu C., et al 2012). The development of highly sophisticated gene transfer methods with better safety and therapeutic efficacy is the consequence of an understanding of the molecular and cellular mechanisms causing treatment- and/or vector-associated failures (Sterry Blunt RE., et al 2015). A number of Phase I/II trials were launched in the last few years using these cutting-edge techniques, with great clinical outcomes and no side effects recorded thus far. Additionally, therapeutically useful site-directed gene editing technologies and extremely effective gene targeting strategies has been created. Gene therapy has evolved from a theory to a clinical reality with more than 1900 clinical trials completed to far (Seng P., et al 2009). This study focuses on the use of gene therapy to treat inherited

diseases, the limitations and issues that some early clinical studies raised, and the resurgence of gene therapy as a potent treatment option for treating monogenic disorders (Ferreira L., et al 2010). Due to considerable advancements in targeting, transport, and safety of gene therapy vectors, gene therapy has matured in many ways. Traditional methods of therapeutic gene delivery using viral and non-viral vectors to make up for defective or deleted genes have been shown in preclinical animal models and clinical trials. Overexpression of immunostimulatory genes has also shown therapeutic benefit in the treatment of cancer. As a form of gene therapy, the delivery of small interfering, short hairpin, and micro-RNA has produced effective gene silencing by RNA interference. Vaccines that prevent infectious diseases and treatments for cancer that stimulate the immune system can also be viewed as gene therapy applications. More modern strategies include CAR-T technology, in which T-cells are modified to produce an artificial T-cell receptor for use in immunotherapy. Furthermore, gene therapy applications have been greatly expanded by CRISPR technologies, making it possible to specifically replace dysfunctional genes with identically working ones (Tevenson LG., et al 2010).

DISCUSSION

Since the gene was identified as the fundamental component of heredity, the capacity to alter the human genome at precise sites has been a goal in medicine. Gene therapy is thus defined as the ability to change a person's genetic makeup through the correction of altered (mutated) genes or site-specific alterations that are intended to treat a medical condition. The development of genetics and bioengineering, which allowed for the manipulation of vectors for the transfer of extrachromosomal material to target cells, made this therapy possible. The optimisation of delivery vehicles (vectors), which are mostly plasmids, nanostructured materials, or viruses, is one of the main areas of concentration of this technique. Due of their prowess at invading cells and introducing their genetic material, viruses are more frequently studied. Exacerbated immunological reactions and genome alteration, particularly in germ line cells, are of major concern. Somatic cell in vivo investigations with approved protocols in clinical trials produced satisfactory findings. These trials have taken place in China, Europe, Australia, and the United States. This article discusses recent developments in biotechnology, including the use of induced pluripotent stem cells in patients with liver illnesses, chimeric antigen receptor T-cell immunotherapy, and genome editing with CRISPR/Cas9.

CONCLUSION

As new insights into important facets of the underlying molecular pathophysiology are gained, therapeutic approaches for gene substitution or addition in neurodegenerative brain illnesses continue to change. Now that iMRI-CED is available, the neurosurgical profession is prepared to maximise the potential translation of these tactics for clinical success. IMRI-CED enables reliable therapeutic vector delivery with real-time verification. Toxic myeloablation and the price of current ex vivo hematopoietic stem cell gene therapy platforms, however, pose obstacles to their widespread use. These gene therapy techniques and ongoing clinical trials are outlined in this study. Finally, we go over potential tactics for enhancing results, minimising myeloablative regimens, and addressing upcoming difficulties in lowering the price of the gene therapy platform. Alternately, genome editing could target particular mutations to restore the expression of the wild-type -globin gene, restoring expression of foetal haemoglobin. The results of the most recent clinical trials for -thalassemia and SCD are encouraging: patients were able to stop receiving transfusions or had lower transfusion needs.

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